

Patient Preference Workshop

Patients and Data Collection

September 18, 2013



PATIENT PREFERENCE AND MEDICAL DEVICES

What Matters?

- ✓ Patients
- ✓ Context
- ✓ Methods
- ✓ B-R Ratio
- ✓ The TPLC
- ✓ Partnerships
- ✓ Risk Communications

Patient Views on Benefits and Risks of Medical Devices

Understanding what patients know about the benefits and risks of medical devices and how they form preferences.

For the online webcast: Please submit your questions to the panel via the chat box. The online hosts will be collecting the questions during the session to be brought to the panel moderator during the panel discussion.

Moderator: ***Randall Brockman, M.D.***
Chief Medical Officer
CDRH/Office of Device Evaluation

Panel: ***Bray Patrick-Lake, M.F.S.***
Clinical Trials Transformation Initiative (CTTI)

Lisa Phelps, M.P.H.
National Organization for Rare Disorders (NORD)

Jennifer Sheridan, M.P.P.
Parkinson's Action Network (PAN)

Margaret Anderson, M.A.
Faster Cures

Marjana Marinac, Pharm.D.
Juvenile Diabetes Research Foundation (JDRF)

Wendy Selig, M.S.
Melanoma Research Alliance (MRA)

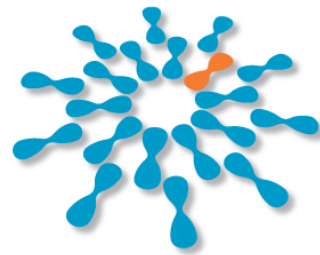
Create tool to give patients a framework for risk/benefit conversation

Treatment Option Comparison

- What is the burden of medical therapy on your life? i.e. side effects, dosing regimen, route of admin.
- Are you refractory to medical therapies?
- Are you out of treatment options?
- Will this device significantly improve the quality of your life?
- If deciding between surgery or transcatheter procedure, what are the risks for infection or bleeding? What is the difference in recovery time or post-procedural disability?
- How comfortable are you with the uncertainties?

Device considerations

- How long will I be using the device?
- Is the device being used off-label? If so, what is known about its use in patients like me?
- Will the device be implanted?
- If implanted, will it have to be replaced?
- What is the long-term performance evidence for device?
- What happens if the device fails?
- Is the device covered by my insurance?



NORD

National Organization for Rare Disorders

The Voice of the Rare Disease Patient Community



Patients in the Rare Disease Community

- Over **7,000** rare diseases affecting approx. 30 million Americans
- Only about **300** rare diseases have an FDA-approved therapy
- Since 1996, over 300 Humanitarian Use Device (HUD) requests
 - **200** designations
 - **55** approved devices for rare diseases and conditions
- Rare Diseases are poorly understood, have relatively poor natural histories and limited research
- Many patients with rare diseases are willing to take on a greater risk to derive medical benefit, especially when no alternate treatment/therapy exists and little information is available
- Anecdotal information to convey their preference for medical devices is crucial to improving access for them to life-saving treatments,

How will patient preference information be used, or provided, to convey evidence of willingness to accept observed or expected risks?



NORD's Key Initiatives:

- Improve access to therapies
- Support innovation & incentives
- Efficient & effective processes for approvals
- Accelerate diagnosis
- Advocate for diseases with no therapies
- Drive the rare disease agenda moving forward



Patient Views on Benefits and Risks of Medical Devices

Jennifer Sheridan, MPP

Director of Policy

September 18, 2013

CDRH Patient Preference Initiative Workshop

Parkinson's Disease Facts

- Between 500,000 and 1.5 million Americans currently have Parkinson's disease
- Parkinson's is the 14th leading cause of death in the United States
- Average age at onset is 60, people have been diagnosed as young as 18
- There is currently no treatment or therapy to stop or slow the progression of Parkinson's
- Symptoms of Parkinson's often include motor and non-motor symptoms:
 - Tremors or trembling, rigidity, slowness of movement, impaired balance and coordination, cognitive changes, difficulty speaking, digestive problems, and sleep disruptions
- Treatments
 - Surgical device treatment: deep brain stimulation (DBS)
 - Multiple symptomatic drug treatments

Risks and Benefits

- Parkinson's is a chronic, progressive, and variable disease
 - Symptoms vary from person to person
 - Ex. Sleep disruptions vs. tremors
- Same diagnosis, different perspectives
 - Newly diagnosed vs. living with the disease for many years
- Not all treatments are of equal value to all patients
- Patient input needed throughout the development and review process

Margaret Anderson, M.A.

Executive Director

Faster Cures

Marjana Marinac, Pharm.D.

JDRF Volunteer

Juvenile Diabetes Research
Foundation (JDRF)

CDRH: An important stakeholder for melanoma patients

Prevention

- Tanning beds

Diagnosis and Staging

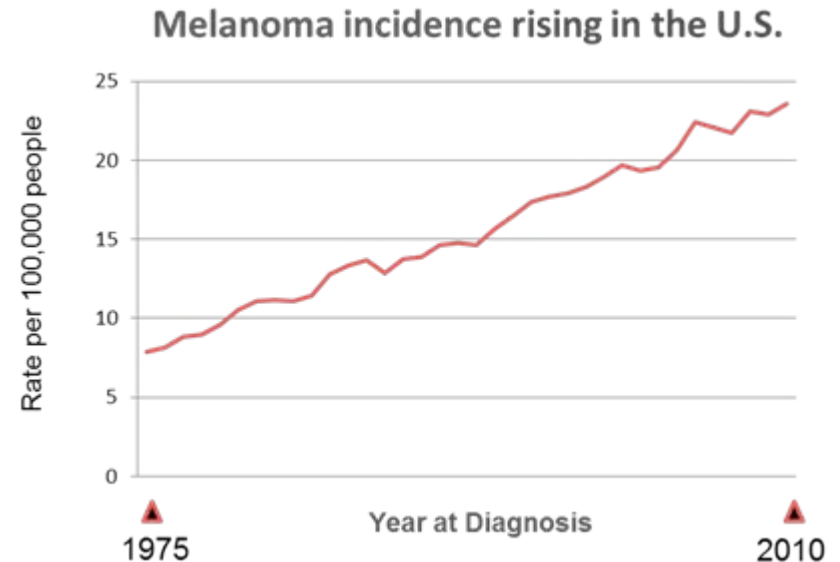
- Skin screening technologies
- Imaging agents
- Prognostic biomarkers

Treatment

- Biomarkers of treatment response/nonresponse
- Companion diagnostics

Melanoma: An ideal case study

- **Need for new treatments:** Aggressive cancer, poor prognosis in late stage, incidence rising dramatically
- **Scientifically and clinically:** At crossroads of molecular biology and immunology
- **New drug approvals:** Prior to 2011, there was little to offer patients with metastatic melanoma -- four newly approved agents since then have changed the landscape for patients
- **Companion Dx and Biomarkers:** Melanoma is leading the way in building this field



**Accelerate development of and
access for patients to
sophisticated tools to define
patient populations and identify
effective biomarkers**

Panel Questions

- How do patients view the benefits and risks of medical devices?
- What kind of information do patients use to form their decisions relating to treatment options?
- What kinds of patient preference information should and should not be collected from patients?
- How should patient preference information be collected?

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